
Site-specific gene editing in hematopoietic stem cells as an anti-HIV therapy

Grant Award Details

Site-specific gene editing in hematopoietic stem cells as an anti-HIV therapy

Grant Type: Tools and Technologies III

Grant Number: RT3-07848

Project Objective: Site-specific gene editing in hematopoietic stem cells (CD34+) as an anti-HIV therapy

Investigator:

Name:	Paula Cannon
Institution:	University of Southern California
Type:	PI

Disease Focus: HIV/AIDS, Infectious Disease

Human Stem Cell Use: Adult Stem Cell

Award Value: \$1,499,400

Status: Active

Progress Reports

Reporting Period: Year 1

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Grant Application Details

Application Title: Site-specific gene editing in hematopoietic stem cells as an anti-HIV therapy

Public Abstract:

The overall goal of this proposal is to develop new methods and technologies to improve our ability to engineer hematopoietic stem cells. These are the adult stem cells found in the bone marrow that give rise to all of the components of the blood and immune systems. Being able to engineer these cells provides potential treatments for diseases of the blood including genetic diseases, such as sickle cell disease or severe immune deficiencies, as well as serious infections such as HIV/AIDS. We work with a new class of genetic engineering tools called targeted nucleases that have the potential to make genetic engineering of stem cells much more precise and therefore safer. In addition, we are exploring methods to deliver these reagents directly to the stem cells in the body, without the currently necessary steps of first removing the cells and performing the genetic engineering in a lab. Such capabilities would greatly improve the safety of human gene therapy, as well as facilitate its practical implementation. HIV/AIDS is our disease of focus, and we will use these techniques to develop new treatments that go beyond the current use of targeted nucleases in patients, where HIV's co-receptor gene, called CCR5, is being disrupted. Our goal is to develop a next-generation of anti-HIV therapies and we expect that the techniques we develop will be broadly applicable to other disease of the blood and immune systems where stem cell therapies could be of benefit.

Statement of Benefit to California:

HIV/AIDS is a major social, economic and health burden to California and its citizens. The numbers are sobering: California has 14% of all US cases of HIV, second only to New York, with 220,543 cases reported through June 2014, including 98,161 deaths. With the advent of improved antiretroviral drugs, mortality has significantly decreased, but so has the length of time people need to take the drugs, and the economic burden to the state is revealed by the cost of drugs representing 85% of all AIDS-related costs. Both federal and state laws require that the AIDS Drug Assistance Program be the payer of last resort for these medications, and its budget is underwritten by the General Fund. Beyond the fiscal concerns, patients live with the potential for developing side effects to the drugs or drug-resistant virus, and accessing these life-long drug regimens is a daily struggle for many. Consequently, the development of stem cell based therapies for HIV brings the potential of one-shot and long-lasting treatments that could arm a patient's own immune system with the capability to suppress HIV in the absence of drugs. Such an outcome would provide economic returns over the long-run by reducing spending on drugs, as well as improving the quality of life for individuals with HIV/AIDS. Beyond HIV, the development of technologies to improve the efficiency, safety and implementation of hematopoietic stem cell therapies will benefit other diseases where such cells could be curative.

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